(MM). **METHODS:** Telephone interviews lasting approximately 1 hour were conducted with two HTA experts from each country, based on an interview guide. Interviews were recorded and common themes identified from assessment of the narrative. **RESULTS:** The French advisors considered that the cost savings realized through informal care compared with formal caregiving are neglected, and that data on informal caregiver burden should be incorporated into cost-effectiveness models where available. The German advisors suggested that caregiver burden may be recognized in HTA if it can be related to patient benefit. Discussions in this area are increasing but changes to the acceptability of caregiver burden data was mostly likely to occur elsewhere in the healthcare system (i.e., outside of the benefit assessment process). Advisors in the US reported that payers' views of caregiver burden are changing and that this information should be considered as more patients involve their caregivers in decision-making, or caregivers make the decisions (for some groups of patients). **CONCLUSIONS:** Informal caregiver burden data are not currently considered important in HTA in Europe or the US, although there are suggestions that this may change. Even where such data have been included in HTA submissions, they have not generally been a driver of HTA decision-making.

PSY133

ASSESSMENT OF REAL-WORLD TREATMENT PATTERNS AND OUTCOMES IN RELAPSED OR REFRACTORY MULTIPLE MYELOMA: EVIDENCE FROM A BRIEF MULTI-COUNTRY SURVEY OF EUROPEAN PHYSICIANS

Davis KL[1], Lin HM[2], Hui A[2], Zhang S[3], Kaye JA[4]

[1]*RTI Health Solutions, Research Triangle Park, NC, USA,* [2]*Millennium Pharmaceuticals, Inc., a wholly owned subsidiary of Takeda Pharmaceutical Company Limited, Cambridge, MA, USA,* [3]*Takeda Pharmaceuticals International, Inc., Deerfield, IL, USA,* [4]*RTI Health Solutions, Waltham, MA, USA*

OBJECTIVES: Data from real-world European settings describing treatment patterns and outcomes in relapsed/refractory multiple myeloma (RRMM) are limited. This study helps address this knowledge gap using a brief physician survey. **METHODS:** Sixty-one physicians treating RRMM were surveyed (November 2014) in France (n=21), Germany (n=20), and the United Kingdom (UK) (n=20). The survey collected physicians' opinions on typical treatment patterns and outcomes of RRMM patients in the relapse/refractory setting (following disease progression during/after first-line therapy). Analyses were descriptive. **RESULTS:** Sixty percent of physicians in France and the UK were haematologists, versus 10% in Germany (where 80% were onco-haematologists). The proportion of patients with high-risk disease based on ISS stage and cytogenetics was 18%-24% across countries. Bortezomib/thalidomide/dexamethasone was the most common induction regimen (42%) for stem cell transplant (SCT)-eligible patients in France, but was unused in Germany and less commonly prescribed in the UK (17%); bortezomib/cyclophosphamide/dexamethasone was the reported induction therapy for 28% of SCT-eligible patients in all countries. Regardless of SCT status, lenalidomide/dexamethasone was the predominant second-line treatment (37%-48%) reported in France and Germany; in the UK, second-/later-line regimens were more varied, with both lenalidomide- and bortezomib-based regimens being reported as common. Second-/later-line therapy duration was generally short, particularly in the UK where 75% of physicians reported a <6-month duration. Disease progression was the top reason for second/later-line discontinuation; other common reasons included toxicity and completion of planned therapy course. For high-risk patients, >75% of physicians reported median survival of <1 year from first relapse. **CONCLUSIONS:** The proportion of RRMM patients with high-risk disease in real-world settings (18%-24%) may exceed that reported in clinical trials (10%-15%). Second-/later-line therapy duration is typically short (<6 months). Survival prospects for RRMM patients remain limited, particularly for high-risk patients. Patient-level studies are needed to better characterize the unmet needs in RRMM signaled by our findings.

PSY134

CHARACTERISTICS ASSOCIATED WITH ANNUAL BLEEDING FREQUENCY AMONG HEMOPHILIA PATIENTS IN THE UNITED STATES

Chen CX[1], Ullman MM[2], Hord JD[3], Kulkarni R[4], Konkle BA[5], Baker JR[6], Riske B[7], Koerper M[8], Lou M[1], Wu J[1], Nichol MB[1]

[1]*University of Southern California, Los Angeles, CA, USA,* [2]*University of Texas at Houston, Gulf States Hemophilia and Thrombophilia Center, Houston, TX, USA,* [3]*Akron Children's Hospital, Akron, OH, USA,* [4]*Michigan State University, East Lansing, MI, USA,* [5]*Bloodworks NW, Seattle, WA, USA,* [6]*University of California, Los Angeles, Los Angeles, CA, USA,* [7]*University of Colorado Hemophilia and Thrombosis Center, Aurora, CO, USA,* [8]*University of California, San Francisco, San Francisco, CA, USA*

OBJECTIVES: Bleeding episodes pose significant economic and quality-of-life (QoL) burdens on persons with hemophilia. This study aims to identify socio-demographic and clinical characteristics associated with annual bleeding frequency (ABF). **METHODS:** Between 2005-2007 and 2009-2012, the Hemophilia Utilization Group Studies Va and Vb, respectively, recruited hemophilia A or B patients from ten geographically diverse Hemophilia Treatment Centers in the United States. Adult patients or parents of children completed a baseline survey that collected information regarding socio-demographics, clinical characteristics, and treatment patterns. During the two-year observation period, bleeding episodes were collected through regular patient follow-ups. ABF was calculated as the annualized sum of patient-reported bleeding episodes. Two-year drug dispensing information was also recorded and annualized. Multivariable linear regressions were used to assess the association of patient variables with ABF. **RESULTS:** Of 477 recruited patients, 317 with complete baseline and utilization data and at least three months of patient follow-up were included. Among included patients, 49.8% were children, 39.7% used prophylaxis, and 63.1% had severe hemophilia. Variables significantly associated with ABF included 37 or older vs. 2-9 years of age (p=0.03), married or with a partner (p=0.03), hemophilia B (p=0.09), mild/moderate hemophilia (p=0.01), prophylaxis (p<0.01), human immunodeficiency virus (HIV) (p<0.01), hepatitis C virus (HCV) (p<0.01), and annual factor usage per kilogram of body weight (p<0.01). Among adults, age in years (p=0.07), no insurance vs. public insurance (p=0.04), mild/moderate hemophilia (p=0.01), HIV (p<0.01), HCV (p=0.02), and factor usage (p=0.02) were significantly associated with ABF. Fewer variables had a significant association with ABF for children compared to adults, including prophylaxis (p<0.01) and factor usage (p=0.01). **CONCLUSIONS:** Variables associated with ABF include socio-demographics, clinical characteristics and factor usage. The preliminary results reinforce the importance of optimizing treatment for individual differences. Future studies should investigate how variations in bleeding outcomes and treatment are associated with healthcare costs.

RESEARCH POSTER PRESENTATIONS – SESSION V

RESEARCH ON METHODS STUDIES

RESEARCH ON METHODS – Clinical Outcomes Methods

PRM1

CART ANALYSIS AS A TOOL TO DETERMINE OPTIMAL TREATMENT INTENSIFICATION TIME IN DIABETES

Watson L[1], Farquhar R[2], Das R[3], Flynn A[2]

[1]*Explore-Epi Ltd, Mattishall, UK,* [2]*Exploristics Ltd, Belfast, UK,* [3]*Merck Sharp & Dohme Limited, Hoddesdon, UK*

OBJECTIVES: A key objective of treating type 2 diabetes mellitus (TD2M) is maintaining glycaemic control, (glycated haemoglobin-HbA1c) with targets set between 6.5%-7.5%. Doctors frequently fail to intensify treatment in uncontrolled patients and a study was designed to assess the long term clinical effects of this inertia. The study involved Classification and Regression Tree (CART) analyses to evaluate the timepoint that intensification should occur to gain glycaemic control. **METHODS:** Incident T2DM patients were identified in the UK CPRD database between 1 January 2000-31 August 2014 and followed for 5 years. Patients initiated on metformin monotherapy and did not achieved HbA1c<7% after 90 days. CART was applied, with Hba1c level as the dependent variable and three explanatory effects: time to intensification class (TTIc), Year and Year by TTIc interaction. For each iteration TTIc was assigned per subject (i.e. those patients with TTI before or after TTI cut-point). The goodness of fit, the Akaike Information Criteria (AIC), was obtained for the model and the optimal cut point was the one resulting in the lowest AIC value. This created two classes of subjects, rapid and delayed TTI, whose HbA1c values were compared **RESULTS:** The model identified that the optimal intensification time was ≤244 days post the first HbA1c≥7% (rapid TTI). 50.7% of patients with rapid TTI achieved HbA1c target<7%, compared to only 14.0% in delayed TTI, despite rapid patients starting with higher average HbA1c levels (8.65%) than delayed patients (7.85%). The effects of rapid intensification caused immediate and maintained reduction in glucose levels, not observed in the delayed group. **CONCLUSIONS:** CART analysis identified the optimal timing for treatment intensification post loss of glycaemic control as ≤244 days. This analytical method could be used in future database studies to aid in group definition by treatment exposure and provide valuable clinical information for physicians.

PRM2

HOW TO CONDUCT ECONOMIC EVALUATIONS OF NEW TREATMENTS FOR ADVANCED CANCER WHEN OVERALL SURVIVAL DATA ARE NOT AVAILABLE? RESULTS FROM A SYSTEMATIC LITERATURE REVIEW

Beauchemin C[1], Lapierre M[1], Letarte N[1], Yelle L[2], Lachaine J[1]

[1]*University of Montreal, Montreal, QC, Canada,* [2]*Centre Hospitalier de l'Université de Montréal – Hôpital Notre-Dame, Montreal, QC, Canada*

OBJECTIVES: Use of surrogate endpoints like progression-free survival (PFS) and time to progression (TTP) instead of overall survival (OS) in clinical trials for advanced cancer remains challenging from a health economic standpoint. This study assessed the use of surrogate endpoints in economic evaluations of anticancer drugs and methodological approaches adopted when reliable OS data are unavailable. **METHODS:** A systematic literature review was conducted to identify economic evaluations of treatments for advanced cancer published between January 2003 and October 2013. Cost-effectiveness and cost-utility analyses expressed in terms of cost per life-year gained and cost per quality-adjusted life-year using a surrogate endpoint as an outcome measure were eligible. Characteristics of selected studies were extracted and comprised: population, treatment of interest, comparator, line-of-treatment, study perspective, and time horizon. Use of surrogate endpoints and methods adopted when OS data were lacking were analyzed. Two reviewers independently selected studies and extracted data. **RESULTS:** In total, 7,219 studies were identified and 100 fulfilled the eligibility criteria. Most included studies assessed the cost-effectiveness of a biological therapy (65%) in the first-line setting (56%) and in the context of advanced non-small cell lung cancer (24%) or advanced breast cancer (22%). Surrogate endpoints mostly used were PFS and TTP, accounting for 92% of included studies. OS data were unavailable for analysis in nearly 25% of economic evaluations. In the absence of OS data, studies most commonly assumed an equal risk of death for all treatment groups. Other methods included use of indirect comparison based on numerous assumptions, use of a surrogate endpoint as a proxy for OS, consultation with clinical experts, and use of OS data associated with different patient populations or treatment-line. **CONCLUSIONS:** Although several approaches are used, there is no consensus method to estimate the cost-effectiveness of new anticancer drugs in the absence of reliable OS data.

PRM3

SELECTION OF STATISTICAL APPROACH IN UNDERSTANDING THE ROLE OF CONTRAST MEDIA IN INPATIENT INTERVENTIONAL CARDIOVASCULAR PROCEDURES

David G[1], Johnson M[2], Lim L[3], Mallow PJ[4]